FDA Accepts Intercept's New Drug Application for OCA for the Treatment of Pre-Cirrhotic Liver Fibrosis Due to NASH

January 19, 2023

NDA is supported by robust NASH clinical development program, including two positive interim analyses from the Phase 3 REGENERATE study demonstrating OCA's improvement in liver fibrosis without worsening of NASH

PDUFA target action date set for June 22, 2023

MORRISTOWN, N.J., Jan. 19, 2023 (GLOBE NEWSWIRE) -- Intercept Pharmaceuticals, Inc. (Nasdaq: ICPT), a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted Intercept's New Drug Application (NDA) for obeticholic acid (OCA) seeking accelerated approval for the treatment of patients with pre-cirrhotic liver fibrosis due to nonalcoholic steatohepatitis (NASH).

FDA indicated that it considers this a complete, Class 2 resubmission and has assigned a Prescription Drug User Fee Act (PDUFA) target action date of June 22, 2023, for the NDA. The timeline for the review of the NDA by FDA remains subject to change.

"This regulatory milestone brings us one step closer to reaching our goal of delivering the first available therapy for patients living with pre-cirrhotic fibrosis due to NASH – the most rapidly growing cause of liver transplantation in the U.S.," said Jerry Durso, President and Chief Executive Officer of Intercept. "We believe OCA has the potential to become an important therapy given its strong and direct antifibrotic effect, and we look forward to continuing our work with FDA over the coming months as they review our NDA."

The NDA is supported by a robust body of evidence from the OCA NASH clinical development program, including two positive, interim 18-month analyses from the pivotal Phase 3 REGENERATE study in patients with pre-cirrhotic liver fibrosis due to NASH. In these analyses, OCA 25 mg consistently demonstrated double the response rate of placebo in reduction in liver fibrosis stage without worsening of any of the three histologic components of NASH, an endpoint consistent with FDA's draft guidance. Further, a detailed assessment of 2,477 patients in REGENERATE, including nearly 1,000 patients on study drug for at least four years, provides a well-characterized safety profile that is monitorable and manageable, and supports chronic administration of OCA.

About the REGENERATE Study

REGENERATE (Randomized Global Phase 3 Study to Evaluate the Impact on NASH with Fibrosis of Obeticholic Acid Treatment) is an ongoing Phase 3, randomized, double-blind, placebo-controlled, multicenter, international study assessing the safety and efficacy of obeticholic acid (OCA) on clinical outcomes in patients with liver fibrosis due to NASH. A pre-specified interim analysis was conducted in 931 subjects who had a liver biopsy at Month 18 to assess the effect of OCA on liver histology as compared to baseline biopsies. REGENERATE is fully enrolled with 2,480 randomized participants and is expected to continue while collecting data on the incidence of clinical outcomes for verification and description of clinical benefit. The end-of-study primary endpoint will compare the impact of treatment group (placebo, OCA 10 mg or OCA 25 mg daily) on all-cause mortality and liver-related clinical outcomes, as well as on long-term safety.

About Liver Fibrosis due to NASH

Nonalcoholic steatohepatitis (NASH) is a serious progressive liver disease caused by excessive fat accumulation in the liver that induces chronic inflammation, resulting in progressive fibrosis (scarring) that can lead to cirrhosis, eventual liver failure, cancer and death. Advanced fibrosis is associated with a substantially higher risk of liver-related morbidity and mortality in patients with NASH. There are currently no medications approved for the treatment of NASH.

About Intercept

Intercept is a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat progressive non-viral liver diseases, including primary biliary cholangitis (PBC), nonalcoholic steatohepatitis (NASH) and severe alcohol-associated hepatitis (sAH). For more information, please visit www.interceptpharma.com or connect with the Company on Twitter and LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements ("FLS"), including regarding the timing of FDA review of our NDA; the prospects for FDA approval of our NDA; the results of our clinical studies; drug efficacy, safety, and tolerability; the commercial opportunity for our product candidate; and the prospects of our product candidate compared to potential competitors. Important factors could cause actual results to differ materially from the FLS. For example, the FDA could take longer than expected to review our NDA; our product candidate could not receive FDA approval in a timely manner or at all; the FDA could require us to provide additional information that is not timely or economical to provide; we could be unable to address to the satisfaction of the FDA the issues raised in its complete response letter of June 2020 responding to our earlier submission; there could be efficacy, safety, or tolerability concerns about our product candidate; our clinical studies could have problems; and our product candidate could have less commercial potential than anticipated or could be superseded by a competing product.

Contact

For more information about Intercept, please contact:

For investors:

Nareg Sagherian, Executive Director, Global Investor Relations
investors@interceptpharma.com

For media:
Karen Preble, Executive Director, Global Corporate Communications
media@interceptpharma.com

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